What is ACTH?

• ACTH is a hormone that is produced in the body.

• Exactly how ACTH works on nephrotic syndrome is unclear.

• ACTH is approved in the U.S. by the F.D.A. to treat nephrotic syndrome.

• The brand of ACTH used in this study is H.P. Acthar Gel.

• H.P. Acthar Gel is given as an injection two times a week.

• For more information on H.P. Acthar Gel, visit: http://www.actharexcessprotein.com.

How to Contact Us:

• Families with a child with nephrotic syndrome or their doctors can contact the study team for more information.

• The study will be explained and if the family is interested in participating, the study team will refer the child to the closest participating medical center.

Study Team

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What is the purpose of the study?

Children with nephrotic syndrome (NS) can have many relapses. They suffer from complications with each relapse. They can also have side effects from NS treatment.

ACTH is effective in treating adults with NS. We want to find out if ACTH is effective for children with NS.

What are the steps involved?

- Candidates will be contacted and meet the study team at the closest participating medical center.
- Eligibility will be determined by the study team. Consent will be obtained for those who agree to participate in the study.
- Participants will be randomized to either ACTH or no ACTH treatment.
  - Participants randomized to ACTH will take ACTH for a total of 12 months.
  - The ACTH dose will be reduced during the second 6 months of treatment.
  - Other medications used to treat NS will be stopped.
  - Participants will be monitored closely for relapses and side effects with clinic visits and telephone calls.
  - Blood work will be obtained at the start of the study, 6 months, and 12 months into the study.
  - Participants randomized to no ACTH will have other medications used to treat NS stopped for up to 6 months.
  - Participants will be monitored closely for relapses and side effects with clinic visits and telephone calls.
  - Blood work will be obtained at the start of the study and 6 months into the study.
  - Participants who relapse will be given the option to start ACTH.

What are the study costs?

- Participants will not be charged for the study lab tests.
- ACTH will be provided free of charge.
- Participants will receive $50 for each study visit to help pay for the costs of travel.

What are the benefits to participating in the study?

- Participants may benefit from the study if they receive ACTH and ACTH prevents NS relapses.
- We do not know if ACTH prevents relapses.
- This study is designed to learn more about the effects of ACTH on NS in children. The results may help other patients in the future.

What are the possible risks and harms?

- Participants may experience more relapses. They will be monitored closely and treated with prednisone for all relapses.
- ACTH has side effects. We may not know all the side effects. Participants will be monitored for side effects. All known side effects will be discussed at the time of consent.

Who is eligible for the study?

- Children and young adults ages 2 to 20 years.
- Patients with NS that goes into remission when treated with steroids.
- Patients with multiple relapses of nephrotic syndrome (“frequently relapsing” or “steroid dependent”).
- Patients cannot be pregnant, have active infections, diabetes, poorly controlled high blood pressure, or cancer.
- Patients must agree to receive ACTH or no ACTH treatment. All patients will eventually have the option of receiving ACTH.

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